Zolgensma (onasemnogene abeparvovec)

Zolgensma is considered medically necessary for one treatment per lifetime for the treatment of spinal muscular atrophy (SMA) in patients less than 2 years of age who meet **ALL** of the following criteria, confirmed with supporting documentation:

I. Criteria for Approval

- Diagnosis of:
  - SMA Type I by a pediatric neurologist with expertise in the diagnosis of SMA; **or**
  - Diagnosis of likely Type I SMA based on the results of SMA newborn screening
- Genetic testing confirmation of bi-allelic deletions or point mutations in the survival motor neuron 1 (SMN1) gene
- Zolgensma is prescribed by a pediatric neurologist with expertise in the treatment of SMA
- SMA-associated symptom onset before 6 months of age
- Member does not have advanced SMA, including but not limited to any of the following:
  - Complete paralysis of limbs; or
  - Invasive ventilation or tracheostomy;
  - Respiratory assistance for 16 or more hours per day (including non-invasive respiratory support) continuously for 14 or more days in the absence of acute reversible illness (excluding perioperative ventilation)
- Prescriber attests that baseline evaluation has been completed and there are no Contraindications, including all black box warnings on the package insert (PI)
- The patient has not previously received gene replacement therapy for SMA
- If the member is on Nusinersen (Spinraza), it will be discontinued prior to administration of Zolgensma
- Prescriber attests that subsequent evaluation and monitoring will be done according to the FDA label

II. Dosing/Administration

Zolgensma must be administered according to the current FDA labeling guidelines for dosage:

- Zolgensma must be administered intravenously at a dose of 1.1×10^{14} vector genomes (vg) per kg of body weight
- Dose to be administered does not exceed 1 kit

III. Length of Authorization

Period of authorization will be for 3 months after initial approval or until 2 years of age, whichever is first. Authorization is for one administration per lifetime.

IV. Investigational Uses

Maryland Medicaid considers Zolgensma investigational and therefore not medically necessary when the criteria above are not met and for all other indications. Repeat administration of Zolgensma is considered investigational. Concomitant use of Zolgensma and Nusinersen (Spinraza) is considered investigational.